SPRING 2013

Rocky Mountain Hemophilia & Bleeding Disorders Association

RMHBDA Newsletter

The Rocky Mountain

RMHBDA is a 501(c)(3) nonprofit organization founded in 2000 and is a chartered chapter of the National Hemophilia Foundation.

Our mission is to improve the quality of care and life for persons with inherited bleeding disorders, including hemophilia and von Willebrand Disease through education, peer support, resources, and referral.

In This Issue

Rocky Mountain Hemophilia & Bleeding Disorders Association

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www.facebook.com/rmhbda

What RMHBDA Means to Us . . .

Dear Brad & RMHBDA Community,

We just wanted to let you know what a great education weekend we had! Thank you for providing such a great experience for all of us. You and the other families made us feel so welcome, more like we have been a part of this organization for years, rather than this event being our first time! For that we are truly grateful. We also want to thank the chapter for covering our travel expenses to make it possible for us to attend Education weekend. We are so happy to have found you and are looking forward to future events with all of you.

Thanks again,

New RMHBDA Family

We Want to Hear from You!

If you would like to have a testimonial about your experience with RMHBDA highlighted in an upcoming newsletter, please email Brad at brad.rmhbda@gmail.com

Family Camp 2013
June 14- 16

Each summer, RMHBDA invites affected families living in Montana and Wyoming to attend a weekend retreat. The weekend is packed full of education, bonding, and fun!

Luccock Park Camp, Livingston, MT (Paradise Valley) For more info, visit www.luccock.org/

For parents and teens, we will have team building programming led by our guest,

Continued on page 3

RMHBDA Education Weekend 2013

A special "Thank you" to our HTC for co-sponsoring our Education Weekend!

University of Colorado Anschutz Medical Campus

***Thank you** to our generous program funders:*

Accredo Health Inc
Axiom Therapeutics
Bayer Healthcare
Baxter Healthcare
Biogen Idec Hemophilia
CSL Behring
CVS Caremark
Grifols
HF Healthcare
Kedrion USA
Novo Nordisk
Octapharma
Pfizer Hemophilia
Walgreen Infusion Services

RMHBDA Education Weekend was held February 22–24 in Bozeman, Montana with 22 families attending — 32 youth and 40 adults. Educational sessions during Education Weekend included: infusion session, Von Willebrand disease, industry/product presentations,

inhibitors, financial advice, how to be a good consumer, healthcare reform and overcoming challenges in dealing with bleeding disorders. All chapter members spent time visiting our exhibitors as they learned more about each company and their products.

RMHBDA welcomed three extraordinary additions to our Board of Directors: Sara Jestrab of Bozeman, Ben Kuss of Bozeman and Brian Schulz of Billings. We are truly grateful to Sara, Ben and Brian for volunteering their time to serve our community!

Everyone enjoyed the chapter trip to the bowling alley. Children enjoyed field trips to the Museum of the Rockies and Spire Climbing Center. Special thanks to Lisa Maxwell and Heidi Hart for assisting with the organization of the event!

RMHBDA Members 2013

Kevin & Jessica Amende

Anne Arthur

Brad & Susan Benne

Ken & Patricia Benne

Forrest & Christy Berg

Ruth Cahill

Jason & Emily Dugan

Jim Ferriter & Lisa Glass

Sharon Ferriter

Ken & Monica Foster

Donald & Beryle Fromm

Chris & Jana Graham

Amanda & Todd Hamper

David Cohenour & Heidi Hart

Bob & Mary Hunter

Sean Jeffrey

Robert, Carol, Kimberly & Sara Jestrab

Rick & Tanya Lasko

Mary Majerus

Lisa & Scott Maxwell

Sally E. McEldery

Linda Reichardt

Jane & Brian Robertson

James & Jodi Rudell

Ed & Ellen Sparks

Thelmar & Margaret Thorson

Brian & Wyn Schulz

Susan & Doug Scott

Spencer & Kaylee Straub

RMHBDA Board of Directors

Lisa Maxwell
President
Great Falls, MT

Chris Graham
Vice President
Billings, MT

Ben Kuss
Treasurer
Bozeman, MT

Jane Robertson
Secretary
Cody, WY

Sara Jestrab
Bozeman, MT

Brian Schulz
Billings, MT

Spencer Straub
Cheyenne, WY

Education Weekend Photos









See many more and larger versions at

www.facebook.com/rmhbda.

From page 1: **Family Camp 2013**

hemophilia leadership expert, Pat Torrey and some time to relax with other families. This is a great opportunity to learn from and share experiences with one another.

We also have many great activities planned for our campers including arts & crafts projects, field games, and educational sessions for children with bleeding disorders and their siblings. Infusion classes will be offered from HTC RN, Sue Geraghty.

Call Brad with any questions at 406.600.2554

We need help organizing!

If you are interested in serving on the Education Weekend committee, please contact Brad at 406.600.2554.

This is your organization!

2013 Mile High Colorado Camp

Save The Date! July 14–19, 2013

Leadership Pre-Camp Retreat
July 12–14, 2013

Forms will be available in mid-March 2013! Stay Tuned!

The Hemophilia and Thrombosis Center (HTC) is proud to once again sponsor the summer camp program at Rocky Mountain Village.

Continued on page 7

Washington Days 2012 Report

A Program of the National Hemophilia Foundation

NHF Advocacy Priorities: Please contact your state senators and members of congress to voice your opinion in regards to important legislative issues that will affect your community. Your voice and willingness to fight for what you believe does make a difference!

There are two federal hemophilia programs that are *critically* important to our community.

Issue 1: HR 460 – The Patients' Access to Treatment Act

Many insurance plans have formularies, which is a list of drugs that are covered by the plan. In addition, plans create separate tiers to set co-payments: generics (Tier I), name brand (Tier II), and off-formulary, brand drugs (Tier III), specialty tier (Tier IV). Insurers set flat co-pays, *e.g.*, $10/$20/$50, for drugs in the first three tiers. Co-payments for specialty tier drugs (Tier IV) are often set at 25–33% of the cost of the drug. Clotting factor is typically placed on the specialty tier. The yearly cost for clotting factor can be $300,000 per year for a person with severe hemophilia, and can exceed $1 million for a person that develops an inhibitor. People with bleeding disorders simply cannot afford to pay this cost!

Insurers require higher patient cost-sharing to reduce use and incentivize patients to choose lower-cost generic alternatives. But, there are no generics for factor!

This bill will increase access to life-saving drugs by removing the burden of excessive cost-sharing, benefiting people with bleeding disorders, and others with high-cost chronic conditions, such as leukemia and lymphoma, multiple sclerosis, rheumatoid and psoriatic arthritis, lupus, primary immunodeficiency diseases, and Crohn's disease.

Issue 2: HRSA Maternal & Child Health Bureau

This bill provides $4.9 million in funding resulting in critical, multi-disciplinary services not usually covered by insurance, such as PT assessment, case management, and social work services. Reduced funding for the hemophilia program at HRSA could result in staffing cuts at HTCs, jeopardizing the quality of care for people with bleeding disorders.

CDC Division of Blood Disorders

CDC provides about $4 million to HTCs for research and surveillance (decreased by $3 million over the last four years) to prevent joint disease and monitor the safety of the blood supply. Reductions to CDC's hemophilia program would jeopardize activities to monitor the safety of the blood supply, improve treatment and prevention strategies, and ensure access to specialized care.

Sean and I had the opportunity to briefly meet with Montana's Senator Jon Tester and new Representative Steve Daines. One benefit of living in states that don't have a large population, is that your voice and opinion are can be extremely powerful! We all share different political opinions, however HR 460 is co-sponored by a Democratic and Republican, and, in my opinion, is a "fairness" issue. I don't know any family that can afford $50,000 or more out-of-pocket for their medicine. I ask you to please contact your congressmen, and ask your representative in congress to co-sponsor HR 460 and ask your senators to please consider introducing companion legislation.

Congress Contact Numbers

Montana

Senator Max Baucus (202) 224-4515
Senator Jon Tester (202) 228-6673
Representative Steve Daines (202) 225-3211

Wyoming

Senator John Barrasso (202) 224-6441
Senator Mike Enzi (202) 224-3424
Representative Cynthia Lummis (202) 225-2311

BAYER HEALTHCARE AND THE HEMOPHILIA COMMUNITY:

Commitment, Leadership *and* Innovation

Bayer HealthCare

Printed in USA KN10001111

Hemophilia Doesn't Stop Hunter Hart from Securing Victories to Help Bozeman Hawks Wrestling Team Go 2-0

By Gidal Kaiser, Chronicle Sports Writer

In order for Bozeman to beat Belgrade and finish out a clean sweep before the Christmas break, all sophomore 215-pounder Hunter Hart had to do was not get pinned or lose by more than eight points.

Hunter faced Belgrade's Bryce Blumenschien in the final match of the Bozeman-Belgrade dual; 18 points worth of forfeits followed. Those 18 points, when added up to what already transpired, handed the hosts a 40-36 win over their close rivals. But there was the final match to play out.

Blumenschien and Hart were knotted at four going into the third period, and Blumenschein took a one-point lead after escaping from the bottom position. Hart took him down, but the Panther escaped with 1 minute, 30 seconds left in the frame for a 6-all score.

The duo tussled and jostled for nearly a full minute. Hart got himself behind Blumenschien, picked up the freshman and dropped the Panther to the mat with 28 seconds left for an 8–6 lead.

With everyone in Bozeman's South Gym screaming and shouting, Hart kept Blumenschein on his stomach until the buzzer to earn the win and seal Bozeman's fourth victory in four matches over two days, including a 39-34 victory over Missoula Sentinel prior to the Belgrade-Bozeman dual. The sophomore went 3-1 in four matches to raise his varsity record to 4–3.

"Just get out there and battle. Make a battle," Hart said. "Make a battle, take it to him, show him we're the Bozeman Hawks and we're tough."

After a scoreless first three minutes, Hart needed a last-second escape at the end of the second period to tie the match with Blumenschien. Smiling, the Hawk said, "Uh, I don't really want to tell you" what his thoughts were heading into the final stanza before adding: "Just get out there and battle even harder."

"I call him 'Big Hunter Hart' because he has a big heart — that was amazing," Bozeman head coach Nate Laslovich said. "For a kid to come out in that kind of environment and come out with a win? Basically, if he didn't give up a major, we win the dual. For him to come and win just shows the type of kid he is. That kid does not want to lose."

up; we knew had to compete with who we had," Hart said. "You compete for your teammates more than yourself."

Do you have a success story to share? We want to celebrate your accomplishments!

Share it with brad at brad.rmhbda@gmail.com

2013 Calendar of Events & Programs

March 2013
- Hemophilia Awareness Month!

April 2013
- 17 World Hemophilia Day:
- 25–27 HFA Annual Symposium

May 2013
- 1 Education Session, Billings
- 2 Education Session, Bozeman
- 14 Walk "Call to Action" Dinner , Billings
- 15 Walk "Call to Action" Dinner , Bozeman
- 16 Walk "Call to Action" Dinner , Helena

June 2013
- 14–16 RMHBDA Family Camp (Luccock Camp Park, Livingston, MT)
- 17 –28 HTC Satellite Clinic, Billings
- 27 –28 HTC Satellite Clinic, Missoula

July 2013
- 12–14 Mile High Summer Camp Leadership Pre-camp Retreat
- 14 – 19 Mile High Summer Camp (Rocky Mountain Village, Empire CO)

August 2013
- TBA Minor League Baseball Night: Helena, Missoula & Billings (TBA)
- 17–18 Walk Kickoff Event, Helena,. Bozeman & Billings *(Tentative)*

September 2013
- 7 Walk for Hemophilia, Bozeman (Pending park availability)

October 2013
- 3–5 NHF Annual Meeting (Anaheim)
- 18–20 TBA Women's Retreat (Chico Hot Springs) (Tentative)

December 2013
- 4–5 RMHBDA Holiday Party (Bozeman & Billings)

Donations

Sharon Ferriter in tribute to Dick Wolstien

Mary R. Brinkley in honor of Andy Brinkley & Family

Sally McElderly in tribute to Travis Parks

Barbara Arnold in tribute to Nick Foster

Thelmar & Margaret Thorsen in honor of Jericho Hugs

New! Now Donate Online with PayPal

If you wish to support RMHBDA with a donation, now you can do so easily and securely online using PayPal, an eBay company. Just visit www.rockymountainhemophilia.org and click "Donate/Join" on the navigation bar. You don't need to have a PayPal account to use this service.

Education Weekend 2013 Raffl Winners…

Marie Frame, Powell, WY

Ravena Howe, Greybull, WY

Lynette Larson, Roundup, MT

Jaxon Stafford, Powell, WY

National Hemophilia Foundation and Rocky Mountain Hemophilia and Bleeding Disorders Association Present

HEMOPHILIA walk '13 SM

Every step makes a difference

National Presenting Sponsor Baxter

Join Us for Our 2nd Annual Montana & Wyoming Walk for Hemophilia!

Come to the Call to Action Walk Meetings

- Billings — May 14, 6 pm
- Helena — May 16, 6 pm
- Bozeman — May 15, 6 pm

These meetings will follow a presentation by Baxter. Dinner is sponsored by Baxter and locations are TBD.

Our "Call to Action" meetings are a great opportunity to get hands-on knowledge about the walk and teams. Here's the kind of helpful information you'll get at our "Call to Action" meetings:

- Receive guidance & support on fundraising from the pros.
- Get inspired by past success stories.
- Learn about starting your team and register your team at our "Call to Action" meetings.
- Summary of "The Power of the Community Voice" presented by Kim Isenberg of Baxter.
- State Advocacy updates and training tools to refresh help us comprehend and influence our leaders to help our community.
- Find out how the chapter staff can support you.
- Discuss the best ways to communicate with team members and motivate them.
- Learn how to lead your team by example.
- Ask questions about the Walk and starting a team.
- Find out about our new online fundraising platform.
- Get info on our Walk Kick Off event & sponsorship.
- Meet and talk with other community members participating in the Walk.
- Have fun... and a free dinner!

The "Call to Action" meeting is great for ANYONE who is interested in starting a team or fundraising for a team. All are welcome... spread the word!

RSVP by Tuesday, May 8 to Brad Benne, Executive Director of RMHBDA if you can join us at 406.586.4050 or brad.rmhbda@gmail.com. If you who are unable to attend the meeting, but would like to participate, please contact Brad to join the Montana and Wyoming Walk for Hemophilia team!

For more information about our Hemophilia Walk and other chapters across the country, visit http://www.hemophilia.org/walk/

From page 3: **2013Mile High Colorado Camp**

Who Should Attend?

- Children with hemophilia or other bleeding disorders
- Siblings of the above groups

Mile High Colorado Camp is for ages 7–18. We accept 6-year-olds on a case by case basis. Programming is determined by age. Check back with us to learn about the different programs we offer at camp!

Why Attend Camp?

The purpose of camp is to learn about bleeding disorders, develop skills and have fun! Campers will have the opportunity to meet new friends and participate in a variety of traditional camp activities. As always, we have included educational components with the goal of encouraging self-confidence and independence.

Many campers have learned to perform self-infusion, experienced teamwork, and discovered new skills during the week of camp. Staff at the Hemophilia & Thrombosis Center(HTC) and Rocky Mountain Village wants this to be a wonderful experience that creates a wealth of fond memories for your camper.

What Does It Cost?

Each family is required to pay a non-refundable $75.00 deposit. The remainder of the camp cost, approximately $1000.00 per camper, is underwritten by other sources. If you have questions or need additional information, please call Brad Benne at 406.600.2554. Scholarship forms are available. Scholarships will be granted on an individual basis..

Industry News

Baxter Healthcare

For the fourth consecutive year, Baxter Healthcare Corporation is sponsoring the Education Advantage (www.thereforyou.com/educationadvantage) scholarship program to help eligible members of the hemophilia community offset the costs of higher education, advance their careers, and pursue their dreams.

Pfi er

Pfizer Hemophilia is excited to announce the launch of the new application period for the Soozie Courter Hemophilia Scholarship Program. Sign up now to be eligible for the Soozie Courter Scholarship. All applications must be received by no later than May 24, 2013. Scholarship winners will be announced in July 2013. For more information or to download an application, please visit: www.HemophiliaVillage.com.

Biogen Idec

Our mission is to empower people with hemophilia to live the lives they choose. We're enabling students with hemophilia to pursue their passions through a wide range of scholarships totaling $50,000 ... including up to $2,500 for vocational/technical and community colleges, and up to $7,000 for colleges/universities. For more information visit www.biogenidechemophilia.com/scholarships

Novo Nordisk

Save the Dates

- **Inhibitor Family Camp**, The Painted Turtle, California, April 19 – 22, 2013
- **Victory Junction**, North Carolina, October 17-20, 2013.

For more information, visit www.InhibitorFamilyCamp.org.

World Hemophilia Day 2013

50 Years of Advancing Treatment for All

The World Federation of Hemophilia (WFH) and the global community have worked together, for the past 50 years, to improve care and treatment for inherited bleeding disorders. World Hemophilia Day 2013 will take on a special significance as we mark '50 Years of Advancing Treatment for All'.

However, most people with hemophilia or other bleeding disorders still do not receive adequate diagnosis, treatment, and management for their condition. It is important that we reflect on where we have been, where we want to go, and that together, we can Close the Gap.

Join together on April 17to mark World Hemophilia Day. Connect with the global online community, on the WFH's Facebook page, and share your hopes and wishes for the next 50 years. To help support your World Hemophilia Day activities, a poster and other materials will be available by January 31. We encourage you to use these materials as part of your World Hemophilia Day activities.

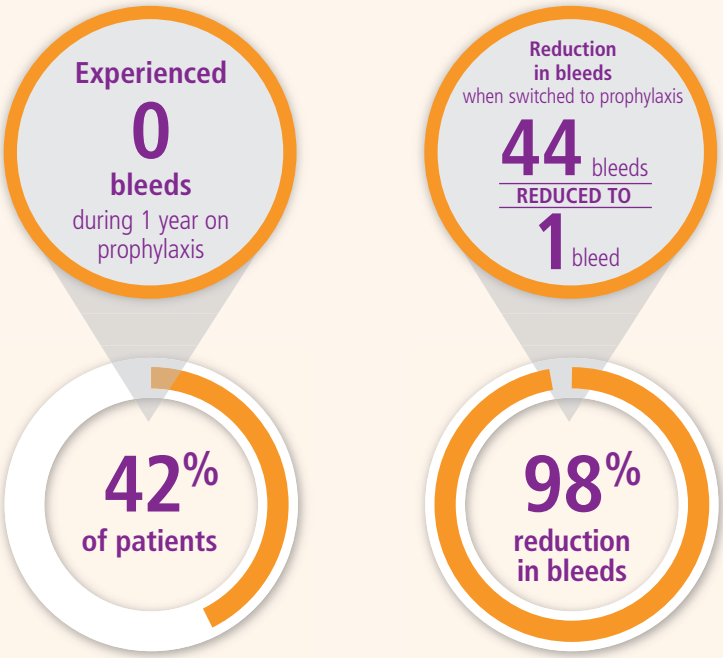
Together, we will Close the Gap. For more information, contact Sarah Ford, WFH communications manager, at sford@wfh.org.

UNLOCKING SELF-POTENTIAL

PROPHYLAXIS WITH ADVATE REDUCED BLEEDS IN A CLINICAL STUDY[1,a]

ADVATE is the only recombinant factor VIII (eight) that is FDA approved for prophylaxis in both adults & children (0-16 years)[1]

Significant reduction in median annual bleed rate (ABR) with prophylaxis treatment compared with on-demand treatment[1,a]



- 0 bleeds experienced by 42% of patients during 1 year on prophylaxis[1,a]
- 98% reduction in median annual bleed rate (ABR) from 44 to 1 when switched from on-demand to prophylaxis[1,a]
- 97% reduction in joint bleeds from 38.7 to 1 after switching from on-demand to prophylaxis[1,a]
- No subject developed factor VIII inhibitors or withdrew due to an adverse event (AE)[2,a]

[a]In a clinical study, after switching from 6 months of on-demand treatment to 12 months of prophylaxis with ADVATE in 53 previously treated patients with severe or moderately severe hemophilia A.

Detailed Important Risk Information for ADVATE

You should not use ADVATE if you are allergic to mice or hamsters or any ingredients in ADVATE.

You should tell your healthcare provider if you have or have had any medical problems, take any medicines, including prescription and non-prescription medicines and dietary supplements, have any allergies, including allergies to mice or hamsters, are nursing, are pregnant, or have been told that you have inhibitors to factor VIII.

You can have an allergic reaction to ADVATE. Call your healthcare provider right away and stop treatment if you get a rash or hives, itching, tightness of the throat, chest pain or tightness, difficulty breathing, lightheadedness, dizziness, nausea, or fainting.

Your body may form inhibitors to factor VIII. An inhibitor is part of the body's normal defense system. If you form inhibitors, it may stop ADVATE from working properly. Consult with your healthcare provider to make sure you are carefully monitored with blood tests for the development of inhibitors to factor VIII.

Side effects that have been reported with ADVATE include: cough, sore throat, unusual taste, abdominal pain, diarrhea, nausea/vomiting, headache, fever, dizziness, hot flashes, chills, sweating, joint swelling/aching, itching, hematoma, swelling of legs, runny nose/congestion, and rash.

Call your healthcare provider right away about any side effects that bother you or if your bleeding does not stop after taking ADVATE.

References:

1. ADVATE Prescribing Information. Westlake Village, CA: Baxter Healthcare Corporation; July 2012. **2.** Valentino LA, Mamonov V, Hellmann A, et al. A randomized comparison of two prophylaxis regimens and a paired comparison of on-demand and prophylaxis treatments in hemophilia A management. *J Thromb Haemost*. 2012;10(3):359-367. **3.** PROLabels: patient-reported outcomes & drug marketing authorizations. ProLabels Web site. http://www.mapi-prolabels.org. Accessed June 5, 2012. **4.** Maruish ME, ed. *User's Manual for the SF-36v2 Health Survey*. 3rd ed. Lincoln, RI: QualityMetric Incorporated; 2011.

Baxter

AND PROVIDED CLINICALLY MEANINGFUL IMPROVEMENTS[b] IN PHYSICAL HEALTH-RELATED QUALITY OF LIFE[1]

ADVATE is the only recombinant factor VIII with physical health-related quality of life results[3,c]

Overall improvement in physical functioning, well-being, general health, and/or energy level, based on Physical Component Score.[4]

Reduced levels of pain and improvements in the limitations of work due to pain.[4]

BODILY PAIN

Improvements in the ability to perform work or other daily activities.[4]

ROLE PHYSICAL

PHYSICAL FUNCTIONING

Changes not observed in limitations to a range of minor and major physical activities.[4]

GENERAL HEALTH

Changes not observed in the views and expectations of overall health.[4]

[b]Clinically meaningful changes were not seen in the mental health-related component score and sub-categories of Mental Health, Role Emotional, Social Functioning, and Vitality.[1]

[c]Based on a list of 29 other drugs and biologics approved with health-related quality of life data in the labeling, as of June 2012.

Indication for ADVATE

ADVATE [Antihemophilic Factor (Recombinant), Plasma/Albumin-Free Method] is a medicine used to replace clotting factor VIII that is missing in people with hemophilia A (also called "classic" hemophilia). ADVATE is used to prevent and control bleeding in adults and children (0-16 years) with hemophilia A. Your healthcare provider may give you ADVATE when you have surgery. ADVATE can reduce the number of bleeding episodes in adults and children (0-16 years) when used regularly (prophylaxis).

ADVATE is not used to treat von Willebrand Disease.

Please see Brief Summary of ADVATE Prescribing Information on the next page.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

Ask your healthcare provider if prophylaxis with ADVATE is right for you.

ADVATE
[Antihemophilic Factor (Recombinant), Plasma/Albumin-Free Method]
There's more to life.

www.advate.com | 888.4.ADVATE

ADVATE
[Antihemophilic Factor (Recombinant), Plasma/Albumin-Free Method]
Brief Summary of Prescribing Information. Please see package insert for full prescribing information.

INDICATIONS AND USAGE

Control and Prevention of Bleeding Episodes

ADVATE [Antihemophilic Factor (Recombinant), Plasma/Albumin-Free Method] is an Antihemophilic Factor (Recombinant) indicated for control and prevention of bleeding episodes in adults and children (0-16 years) with Hemophilia A.

Perioperative Management

ADVATE is indicated in the perioperative management in adults and children (0-16 years) with Hemophilia A.

Routine Prophylaxis

ADVATE is indicated for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adults and children (0-16 years) with Hemophilia A.

ADVATE is not indicated for the treatment of von Willebrand disease.

CONTRAINDICATIONS

Known anaphylaxis to mouse or hamster protein or other constituents of the product.

WARNINGS AND PRECAUTIONS

Anaphylaxis and Hypersensitivity Reactions

Allergic-type hypersensitivity reactions, including anaphylaxis, are possible and have been reported with ADVATE. Symptoms have manifested as dizziness, paresthesias, rash, flushing, face swelling, urticaria, dyspnea, and pruritus. *[See Patient Counseling Information (17) in full prescribing information]*

ADVATE contains trace amounts of mouse immunoglobulin G (MuIgG): maximum of 0.1 ng/IU ADVATE and hamster proteins: maximum of 1.5 ng/IU ADVATE. Patients treated with this product may develop hypersensitivity to these non-human mammalian proteins.

Discontinue ADVATE if hypersensitivity symptoms occur and administer appropriate emergency treatment.

Neutralizing Antibodies

Carefully monitor patients treated with AHF products for the development of Factor VIII inhibitors by appropriate clinical observations and laboratory tests. Inhibitors have been reported following administration of ADVATE predominantly in previously untreated patients (PUPs) and previously minimally treated patients (MTPs). If expected plasma Factor VIII activity levels are not attained, or if bleeding is not controlled with an expected dose, perform an assay that measures Factor VIII inhibitor concentration. *[See Warnings and Precautions (5.3) in full prescribing information]*

Monitoring Laboratory Tests

The clinical response to ADVATE may vary. If bleeding is not controlled with the recommended dose, determine the plasma level of Factor VIII and administer a sufficient dose of ADVATE to achieve a satisfactory clinical response. If the patient's plasma Factor VIII level fails to increase as expected or if bleeding is not controlled after the expected dose, suspect the presence of an inhibitor (neutralizing antibodies) and perform appropriate tests as follows:

- Monitor plasma Factor VIII activity levels by the one-stage clotting assay to confirm the adequate Factor VIII levels have been achieved and maintained when clinically indicated. *[See Dosage and Administration (2) in full prescribing information]*
- Perform the Bethesda assay to determine if Factor VIII inhibitor is present. If expected Factor VIII activity plasma levels are not attained, or if bleeding is not controlled with the expected dose of ADVATE, use Bethesda Units (BU) to titer inhibitors.
  - If the inhibitor titer is less than 10 BU per mL, the administration of additional Antihemophilic Factor concentrate may neutralize the inhibitor and may permit an appropriate hemostatic response.
  - If the inhibitor titer is above 10 BU per mL, adequate hemostasis may not be achieved. The inhibitor titer may rise following ADVATE infusion as a result of an anamnestic response to Factor VIII. The treatment or prevention of bleeding in such patients requires the use of alternative therapeutic approaches and agents.

ADVERSE REACTIONS

The serious adverse drug reactions (ADRs) seen with ADVATE are hypersensitivity reactions and the development of high-titer inhibitors necessitating alternative treatments to Factor VIII.

The most common ADRs observed in clinical trials (frequency ≥ 10% of subjects) were pyrexia, headache, cough, nasopharyngitis, vomiting, arthralgia, and limb injury.

Clinical Trial Experience

Because clinical trials are conducted under widely varying conditions, adverse reaction rates observed in the clinical trials of a drug cannot be directly compared to rates in clinical trials of another drug and may not reflect the rates observed in clinical practice.

ADVATE has been evaluated in five completed studies in previously treated patients (PTPs) and one ongoing study in previously untreated patients (PUPs) with severe to moderately severe Hemophilia A (Factor VIII ≤ 2% of normal). A total of 234 subjects have been treated with ADVATE as of March 2006. Total exposure to ADVATE was 44,926 infusions. The median duration of participation per subject was 370.5 (range: 1 to 1,256) days and the median number of exposure days to ADVATE per subject was 128.0 (range: 1 to 598).[1]

The summary of adverse reactions (ADRs) with a frequency ≥ 5% (defined as adverse events occurring within 24 hours of infusion or any event causally related occurring within study period) is shown in Table 1.

No subject was withdrawn from a study due to an ADR. There were no deaths in any of the clinical studies.

IMMUNOGENICITY

The development of Factor VIII inhibitors with the use of ADVATE was evaluated in clinical studies with pediatric PTPs (< 6 years of age with > 50 Factor VIII exposures) and PTPs (≥ 10 years of age with > 150 Factor VIII exposures). Of 198 subjects who were treated for at least 10 exposure days or on study for a minimum of 120 days, 1 adult developed a low-titer inhibitor (2.0 [BU] in the Bethesda assay) after 26 exposure days. Eight weeks later, the inhibitor was no longer detectable, and *in vivo* recovery was normal at 1 and 3 hours after infusion of another marketed recombinant Factor VIII concentrate. This single event results in a Factor VIII inhibitor frequency in PTPs of 0.51% (95% CI of 0.03 and 2.91% for the risk of any Factor VIII inhibitor development).[1,2] No Factor VIII inhibitors were detected in the 53 treated pediatric PTPs.

In clinical studies that enrolled previously untreated subjects (defined as having had up to 3 exposures to a Factor VIII product at the time of enrollment), 5 (20%) of 25 subjects who received ADVATE developed inhibitors to Factor VIII.[1] Four patients developed high titer (> 5 BU) and one patient developed low-titer inhibitors. Inhibitors were detected at a median of 11 exposure days (range 7 to 13 exposure days) to investigational product.

Immunogenicity also was evaluated by measuring the development of antibodies to heterologous proteins. 182 treated subjects were assessed for anti-Chinese hamster ovary (CHO) cell protein antibodies. Of these patients, 3 showed an upward trend in antibody titer over time and 4 showed repeated but transient elevations of antibodies. 182 treated subjects were assessed for mulgG protein antibodies. Of these, 10 showed an upward trend in anti-mulgG antibody titer over time and 2 showed repeated but transient elevations of antibodies. Four subjects who demonstrated antibody elevations reported isolated events of urticaria, pruritus, rash, and slightly elevated eosinophil counts. All of these subjects had numerous repeat exposures to the study product without recurrence of the events and a causal relationship between the antibody findings and these clinical events has not been established.

Of the 181 subjects who were treated and assessed for the presence of anti-human von Willebrand Factor (VWF) antibodies, none displayed laboratory evidence indicative of a positive serologic response.

Post-Marketing Experience

The following adverse reactions have been identified during post-approval use of ADVATE. Because these reactions are reported voluntarily from a population of uncertain size, it is not always possible to reliably estimate their frequency or establish a causal relationship to drug exposure.

Among patients treated with ADVATE, cases of serious allergic/hypersensitivity reactions including anaphylaxis have been reported and Factor VIII inhibitor formation (observed predominantly in PUPs). Table 2 represents the most frequently reported post-marketing adverse reactions as MedDRA Preferred Terms.

Table 1
Summary of Adverse Reactions (ADRs)[a] with a Frequency ≥ 5% in 234 Treated Subjects[b]

MedDRA[c] System Organ Class	MedDRA Preferred Term	Number of ADRs	Number of Subjects	Percent of Subjects
General disorders and administration site conditions	Pyrexia	78	50	21
Nervous system disorders	Headache	104	49	21
Respiratory, thoracic and mediastinal disorders	Cough	75	44	19
Infections and infestations	Nasopharyngitis	61	40	17
Gastrointestinal disorders	Vomiting	35	27	12
Musculoskeletal and connective tissue disorders	Arthralgia	44	27	12
Injury, poisoning and procedural complications	Limb injury	55	24	10
Infections and infestations	Upper respiratory tract infection	24	20	9
Respiratory, thoracic and mediastinal disorders	Pharyngolaryngeal pain	23	20	9
Respiratory, thoracic and mediastinal disorders	Nasal congestion	24	19	8
Gastrointestinal disorders	Diarrhea	24	18	8
Gastrointestinal disorders	Nausea	21	17	8
General disorders and administration site conditions	Pain	19	17	8
Skin and subcutaneous tissue disorders	Rash	16	13	6
Infections and infestations	Ear infection	16	12	5
Injury, poisoning and procedural complications	Procedural pain	16	12	5
Respiratory, thoracic and mediastinal disorders	Rhinorrhea	15	12	5

[a] ADRs are defined as all Adverse Events that occurred (a) within 24 hours after being infused with investigational product or (b) all Adverse Events assessed related or possibly related to investigational product or (c) Adverse Events for which the investigator's or sponsor's opinion of causality was missing or indeterminate.

[b] The ADVATE clinical program included 234 treated subjects from 5 completed studies in PTPs and 1 ongoing study in PUPs as of 27 March 2006.

[c] MedDRA version 8.1 was used.

Table 2
Post-Marketing Experience

Organ System [MedDRA Primary SOC]	Preferred Term
Immune system disorders	Anaphylactic reaction[a] Hypersensitivity[a]
Blood and lymphatic system disorders	Factor VIII inhibition
General disorders and administration site conditions	Injection site reaction Chills Fatigue/Malaise Chest discomfort/pain Less-than-expected therapeutic effect

[a] These reactions have been manifested by dizziness, paresthesias, rash, flushing, face swelling, urticaria, and/or pruritus.

References: 1. Shapiro A, Gruppo R, Pabinger I et al. Integrated analysis of safety and efficacy of a plasma- and albumin-free recombinant factor VIII (rAHF-PFM) from six clinical studies in patients with hemophilia A. Expert Opin Biol Ther 2009 9:273-283. **2.** Tarantino MD, Collins PW, Hay PW et al. Clinical evaluation of an advanced category antihaemophilic factor prepared using a plasma/albumin-free method: pharmacokinetics, efficacy, and safety in previously treated patients with haemophilia A. Haemophilia 2004 10:428-437.

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Leaders in Hemophilia Advocacy, Healthcare Delivery and Biotechnology Partner to Offer Free Genetic Testing to People with Hemophilia

- *– New partnership will address cost, the primary barrier to widespread testing*
- *– Initiative will advance hemophilia research by enhancing national database*
- *– Information from new program can also assist in individualizing care in hemophilia –*

ORLANDO, FL – November 8, 2012 – A coalition of leaders in hemophilia advocacy and treatment today announced a nationwide program that will offer free genetic testing to people with hemophilia and their families. The partnership will be officially unveiled this evening at the 64th National Hemophilia Foundation (NHF) Annual Meeting in Orlando, Florida.

The initiative, called My Life, Our Future: Genotyping for Progress in Hemophilia, is a partnership of the NHF, the American Thrombosis and Hemostasis Network (ATHN), Puget Sound Blood Center (PSBC), and Biogen Idec (NASDAQ: BIIB). It is designed to help uncover genetic information that can be used by physicians to individualize the care of people with hemophilia, as well as generate data that may lead to new scientific discoveries.

Genetic testing, or genotyping, is used to identify the genetic mutation that causes hemophilia, a rare inherited disorder that impairs blood clotting. Research has shown that certain mutations are associated with an increased risk for severe bleeding and inhibitors, a major complication that involves an immune response to treatment. Healthcare providers can tailor treatment to the individual needs of patients when these risks are understood. In addition, women in a patient's family are often tested to determine if they carry the gene for hemophilia, and genetic counseling can inform family planning and pregnancy care.

"This partnership is about helping people affected by hemophilia gain a better understanding of their genetic make-up and working with healthcare providers to use that information to provide more individualized care tailored to the needs of families," said Val Bias, Chief Executive Officer of NHF.

Program Will Address Barriers to Genotyping

Despite the potential benefits, only about 20 percent of people with hemophilia in the U.S. have been genotyped, according to a survey of healthcare providers conducted by ATHN. In addition, the U.S. lags behind other developed countries such as Australia and the U.K., which offer genotyping to people with hemophilia as a matter of standard care.

In a recent NHF survey of the hemophilia community, cost and insurance coverage restrictions were cited as primary barriers to genetic testing by people who did not know their genotype. Overall, more than 75 percent of survey respondents expressed a strong interest in receiving free or very low cost genotyping through this new initiative.

"We hope that many people will participate in the My Life, Our Future program to learn more about their genetic profile and to contribute knowledge that may help future generations," said Barbara Konkle, M.D., Director of Clinical and Translational Research at PSBC. "As a physician, I believe this initiative will provide Hemophilia Treatment Centers with a critical tool to individualize care for people with hemophilia and their families today."

National Database and Research Repository Will Be Enhanced

Beyond receiving their individual genotyping results, participants can help accelerate scientific research in hemophilia by contributing data and samples to a secure national database and research repository. In the future, scientists can apply through ATHN for access to the data or samples to conduct research.

The confidentiality of participants who contribute their data and samples will be protected. Patient identifying information will not be entered into the database and the repository, nor given to researchers.

"With an enhanced database, researchers will be able to investigate the connection between the genetic cause of hemophilia and clinical outcomes," said Diane Aschman, President and Chief Executive Officer of ATHN. "Future researchers will be better equipped to improve the care of hemophilia and identify new treatment approaches."

A Partner Dedicated to Serving the Hemophilia Community

Biogen Idec Hemophilia, a business unit of Biogen Idec with expertise in hemophilia and genetic medicine, will provide financial backing for the initiative, as well as scientific advice and project management support.

"Our goal is to empower people with hemophilia to live the lives they choose," said Glenn Pierce, M.D., Ph.D., Chief Medical Officer of Biogen Idec Hemophilia. "This initiative fits perfectly with our mission because it offers knowledge to patients, their families, healthcare providers and researchers that can improve care and advance science."

The company is supporting My Life, Our Future as a service to the community, and will not have special access to the data or samples generated by the initiative.

About My Life, Our Future: Genotyping for Progress in Hemophilia

My Life, Our Future is a partnership of the National Hemophilia Foundation (NHF), . . .

Continue reading on the National Hemophilia Foundation website http://goo.gl/ABGVh

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